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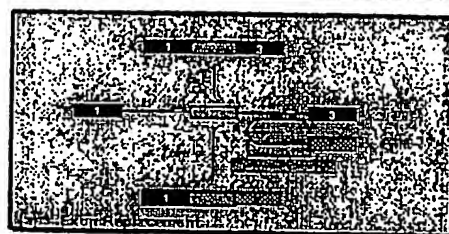
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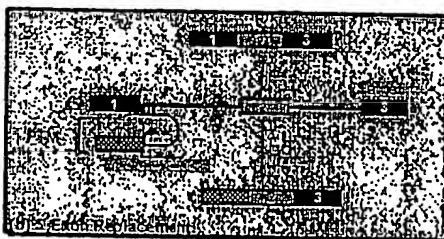


FIGURE 1

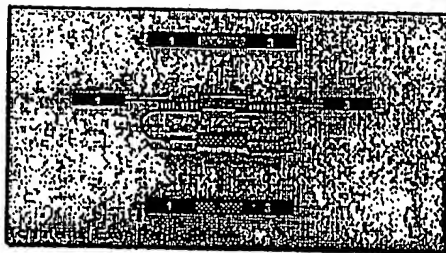
Different SMaRT Reactions



3' ER



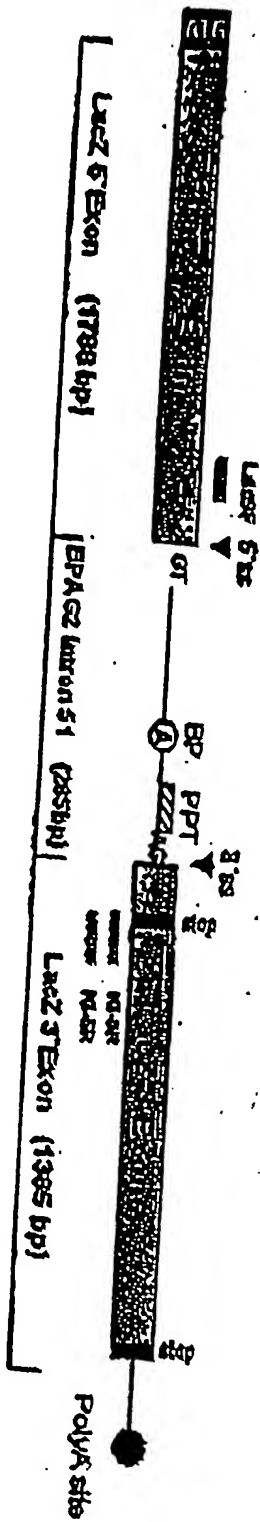
5' ER



Internal

1) BPAG2 Target 1 (LacZ-T1)

FIGURE 2A



2) BPAG2 Target 2 (T2)

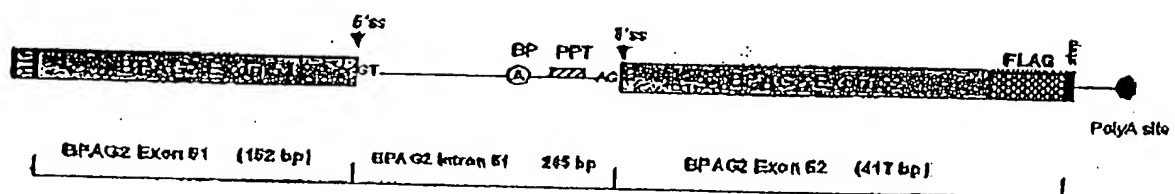


FIGURE 2B

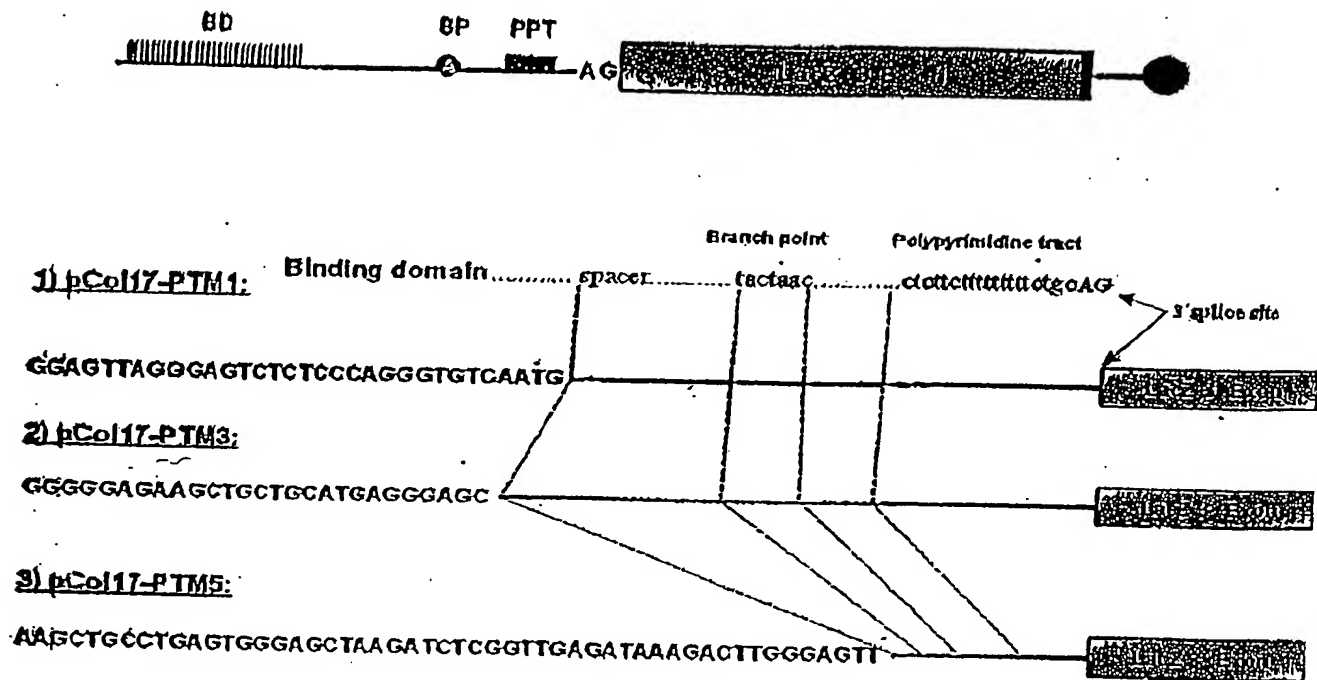


FIGURE 2C

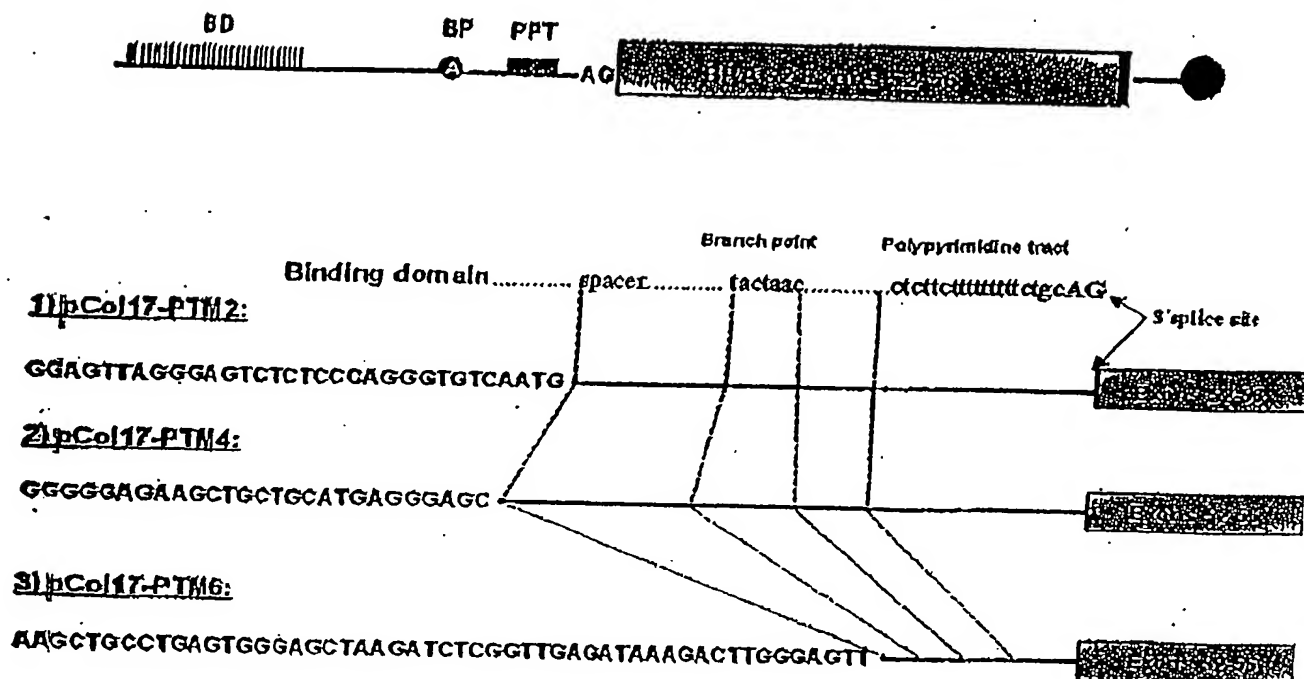


FIGURE 2D

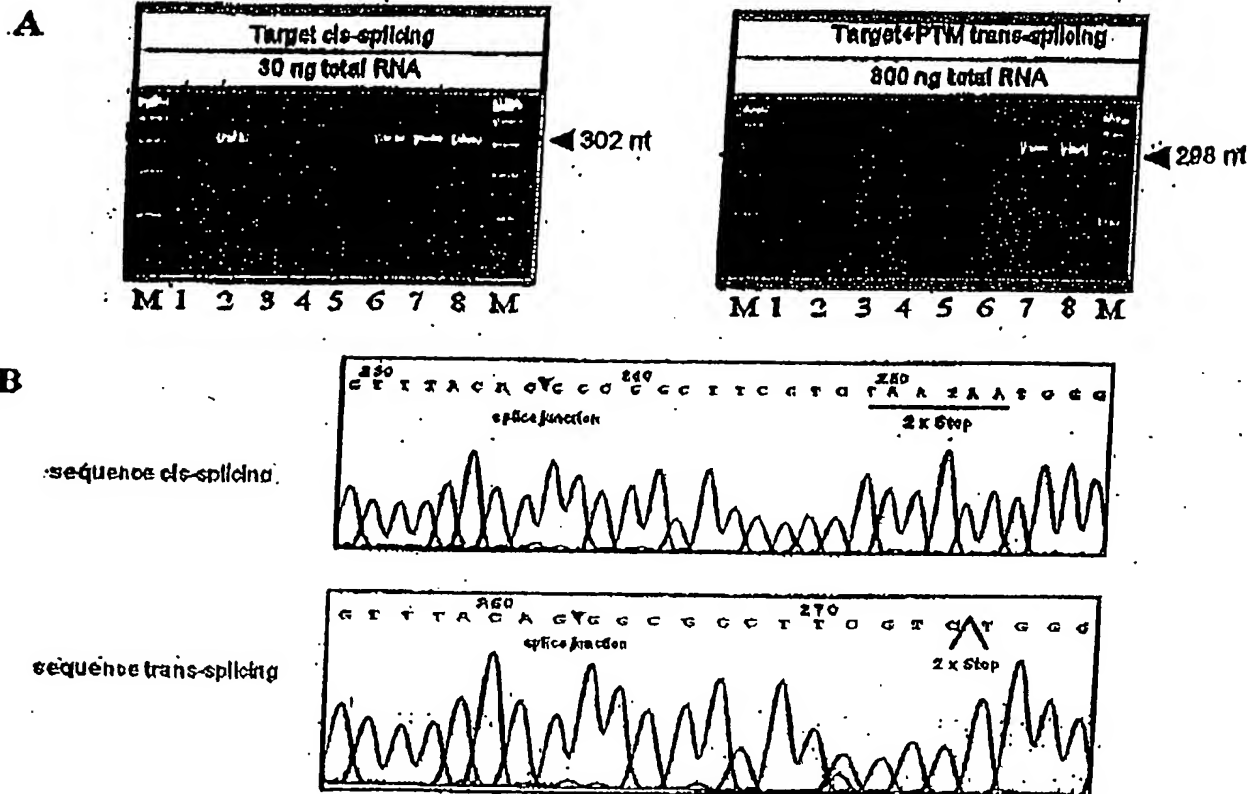


FIGURE 3A-B

Q

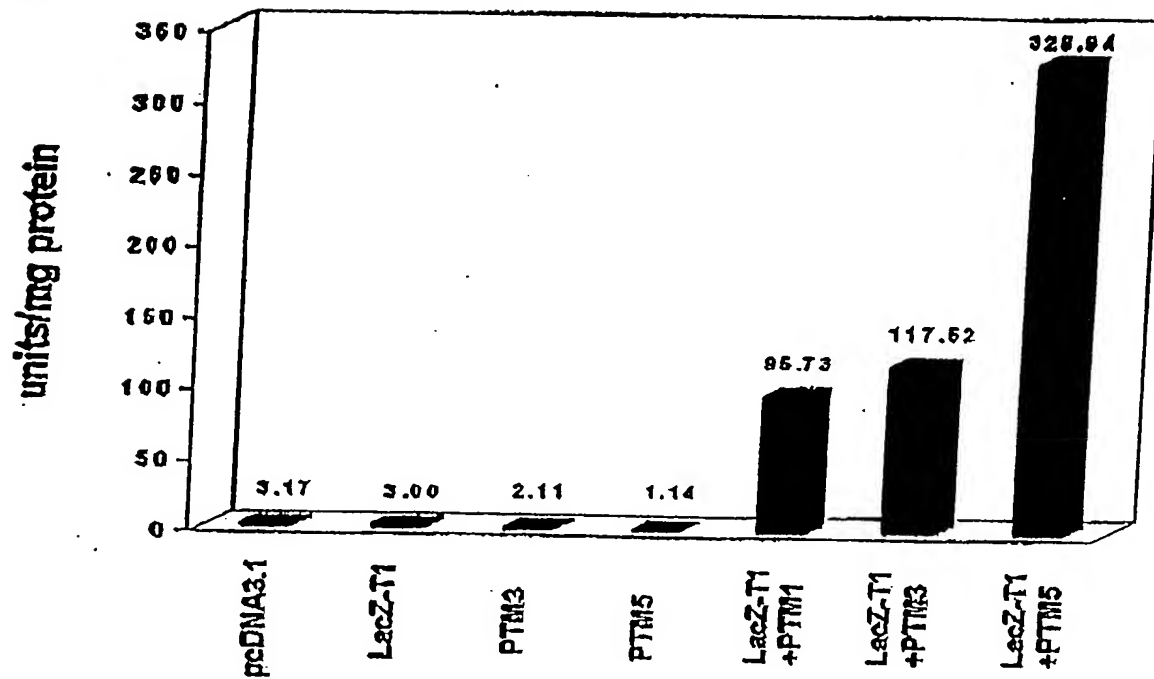


FIGURE 3C

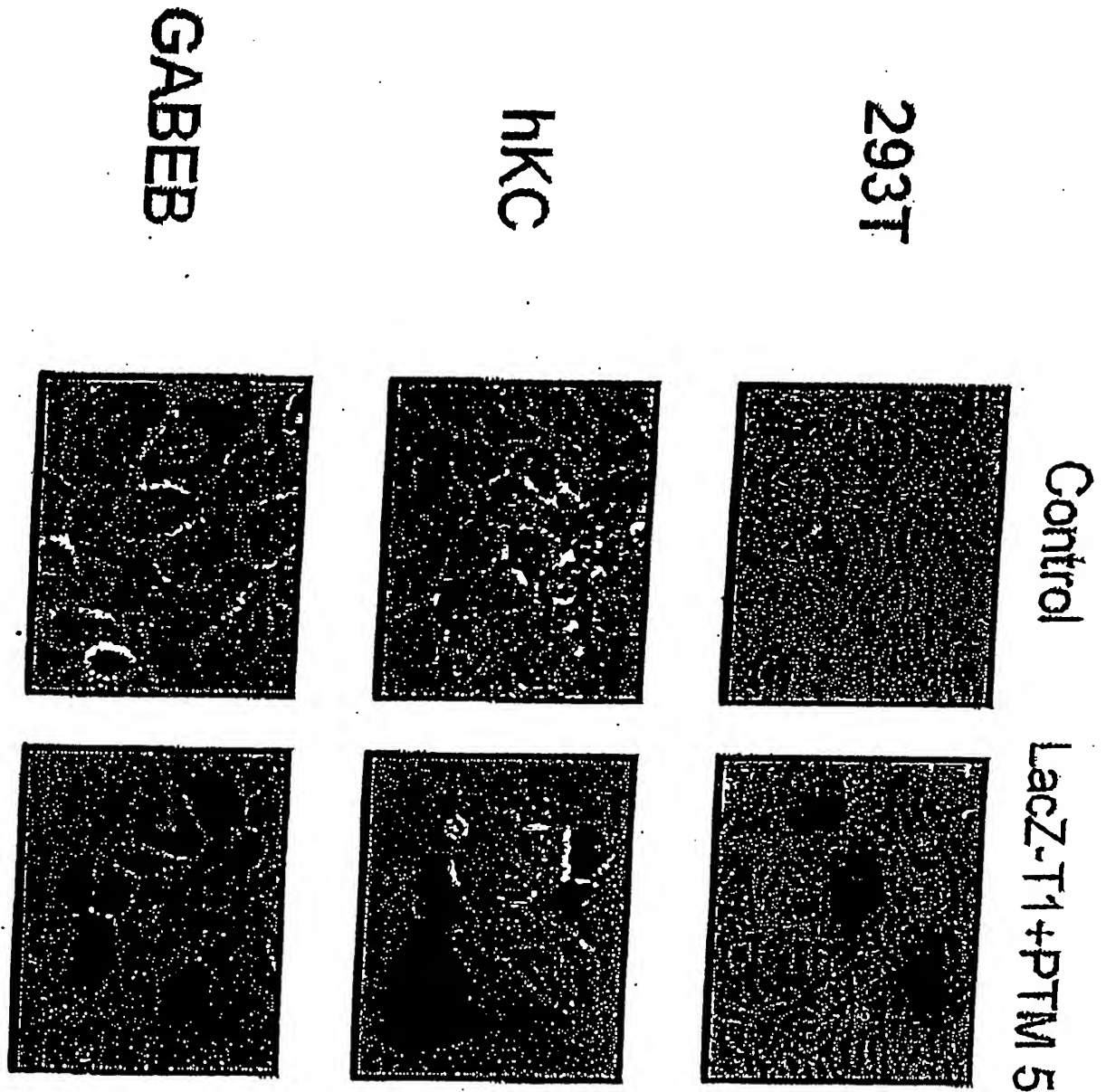
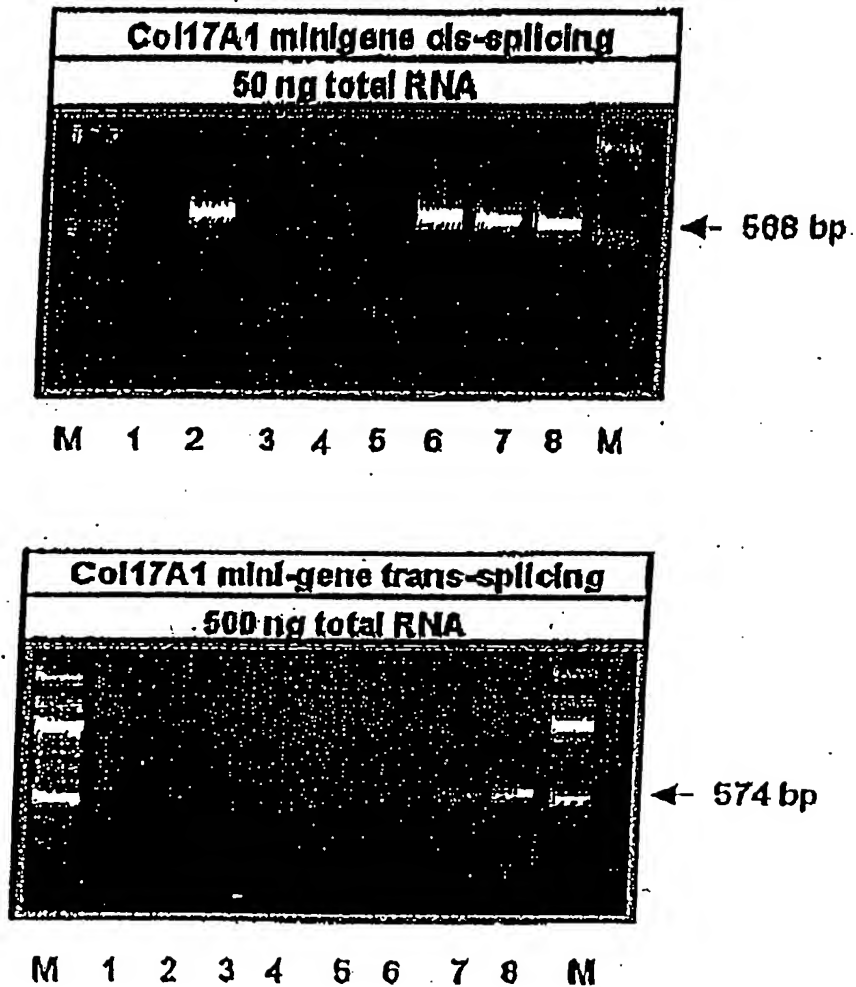


FIGURE 4

A



B

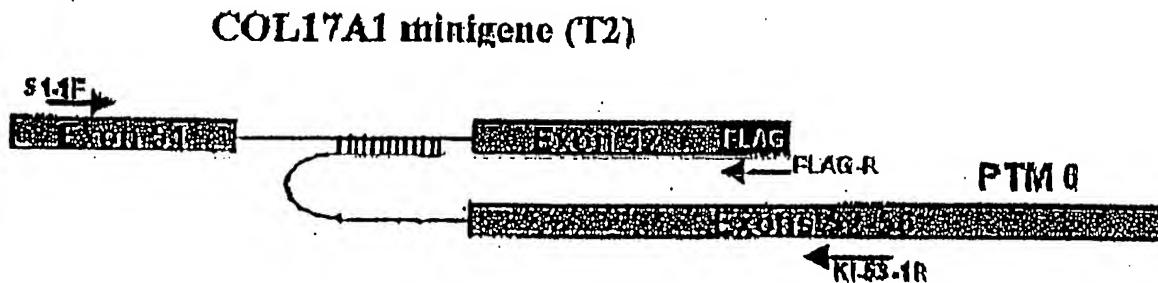


FIGURE 5A-B

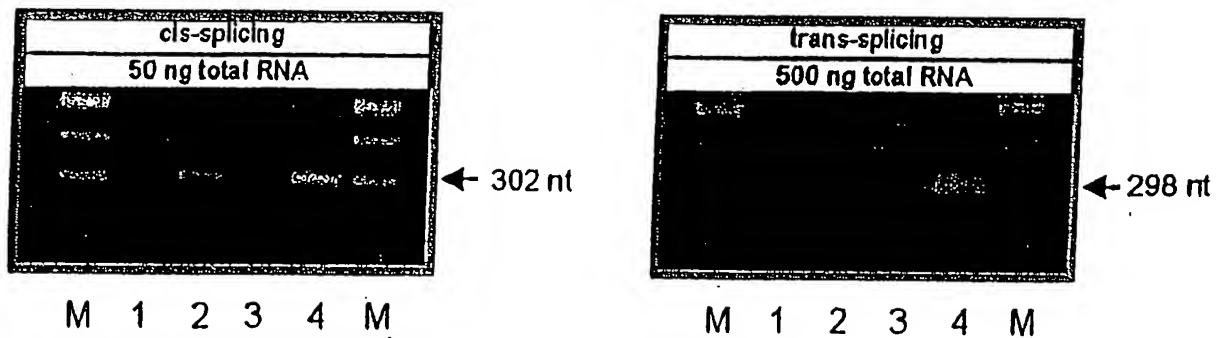
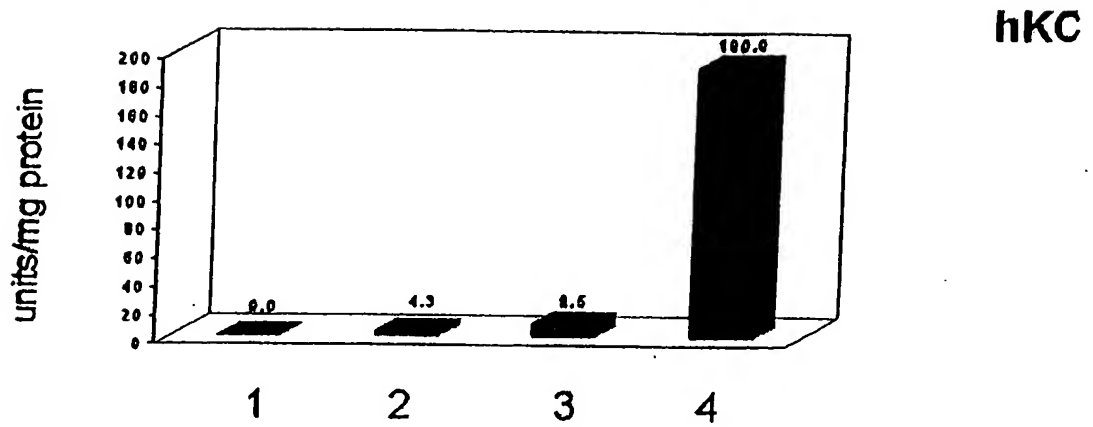


FIGURE 6A

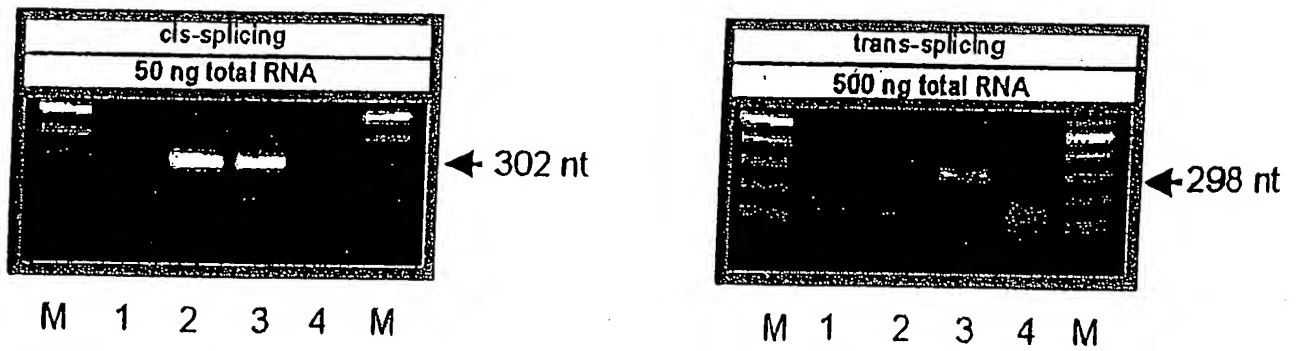
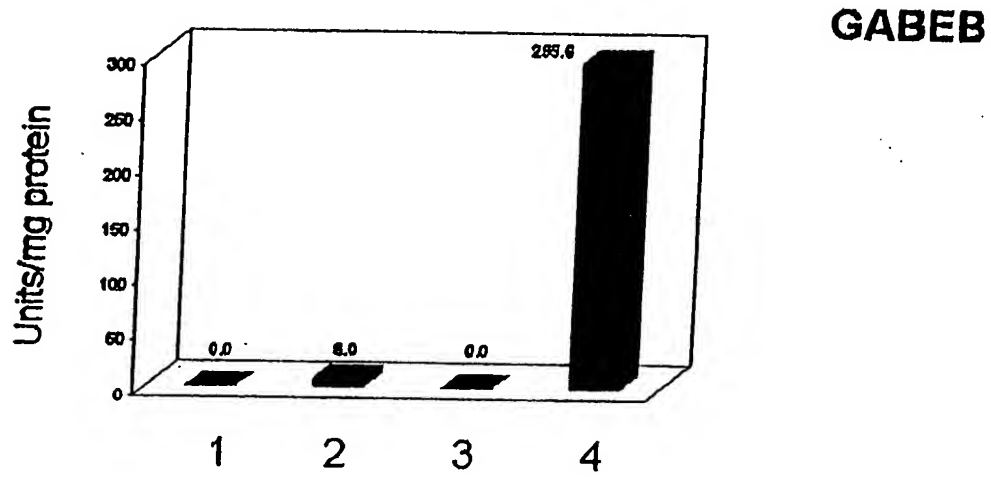


FIGURE 6B

FIGURE 7

Detection Strategy for
 Endogenous Trans-splicing in the
 COL17A1 pre-mRNA in HEK293

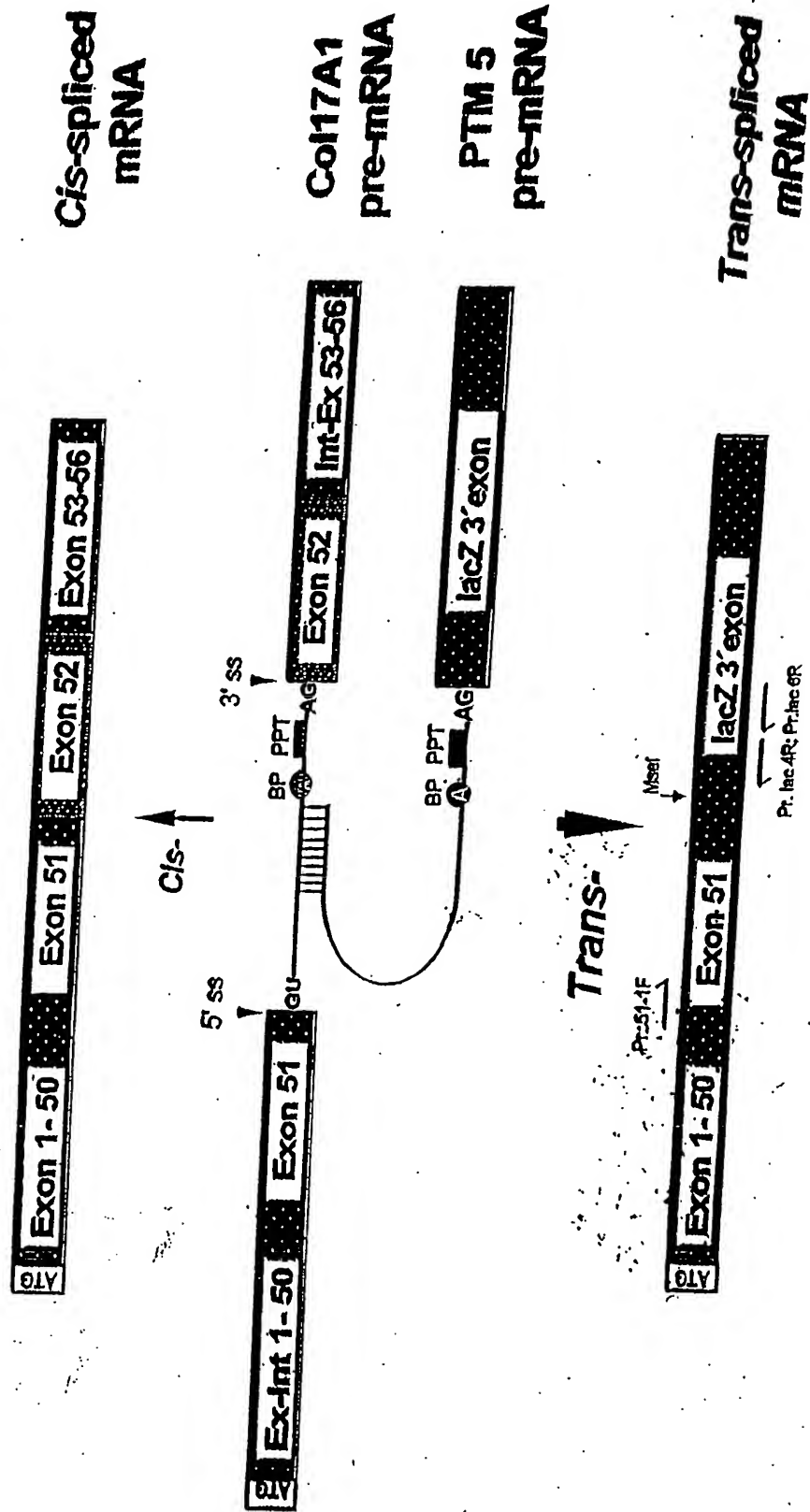
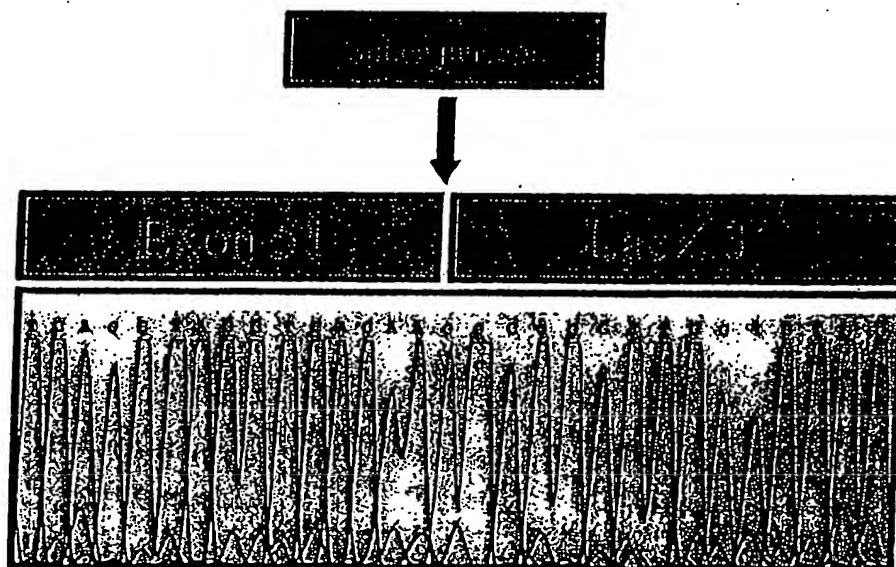


FIGURE 8

Endogenous Transsplicing of Col17 pre-mRNA with PTM5 pre-mRNA

A)



B)

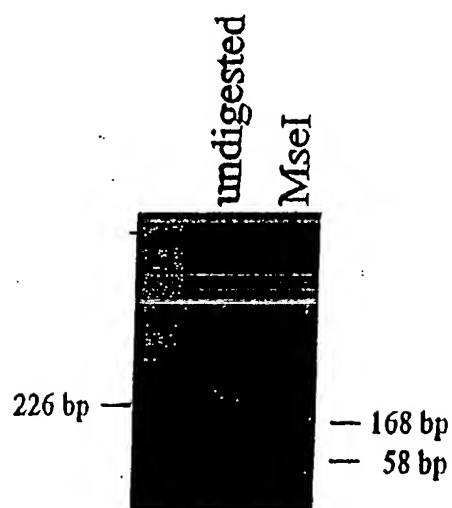


FIGURE 9

5' trans-splicing in a LacZ-model system for hereditary diseases

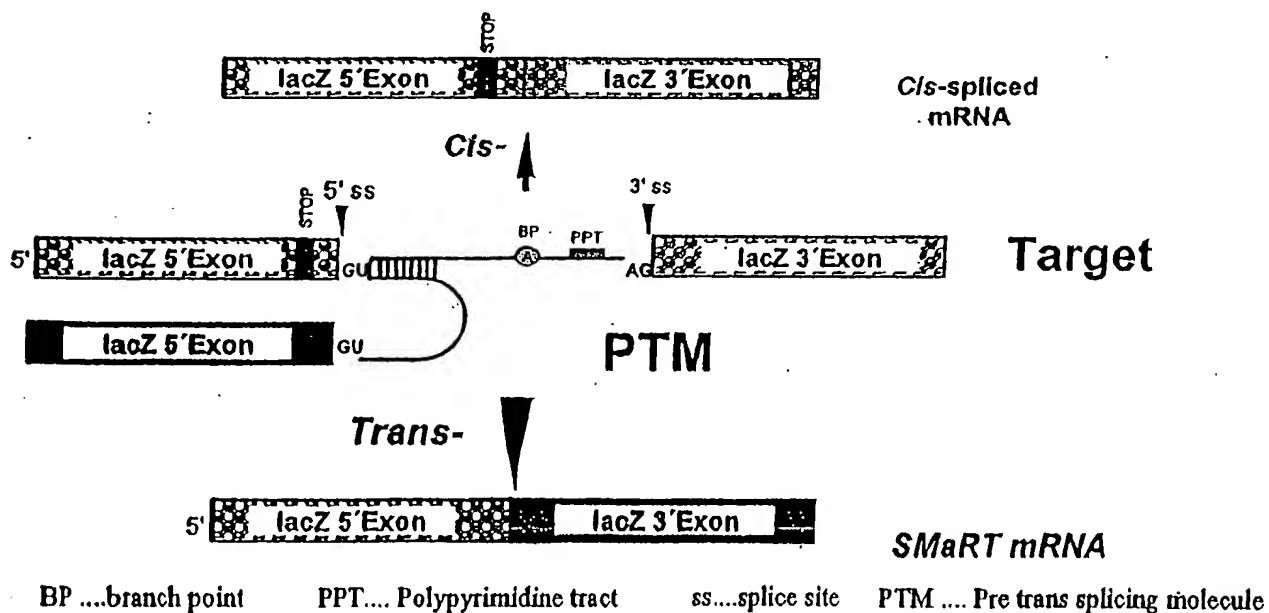


FIGURE 10

Target LacZ-T3 (containing Intron 9) and LacZ-T4 used for optimizing trans-splicing and transfection conditions

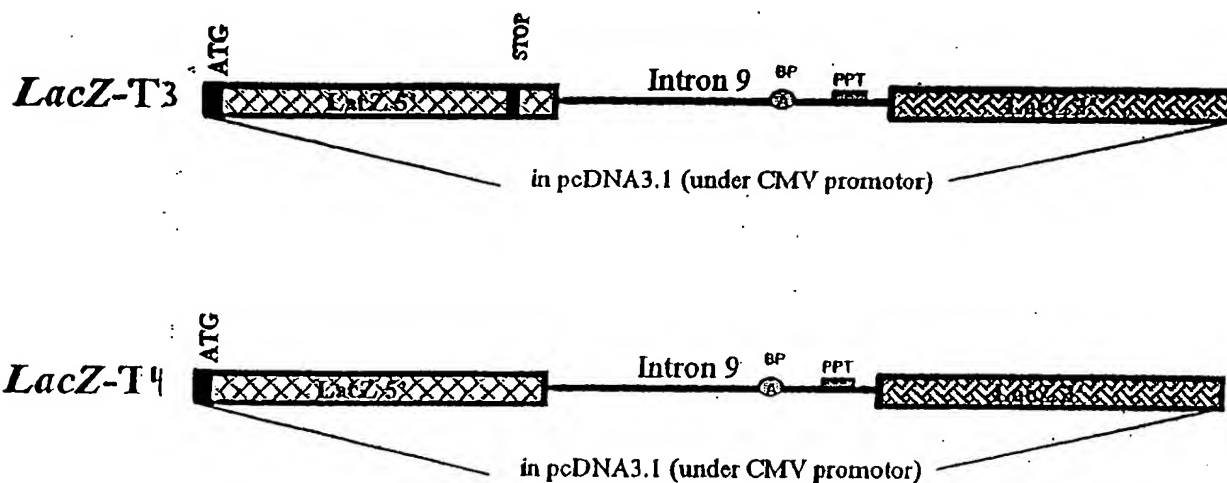
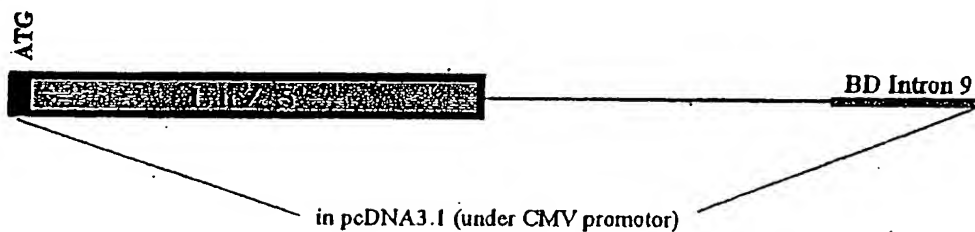


FIGURE 11

LacZ-PTM-3 (Intron 9 specific binding domain) and LacZ-PTM-4 (non specific binding domain) for establishing optimal trans-splicing conditions

Lac-PTM³



Lac-PTM⁴

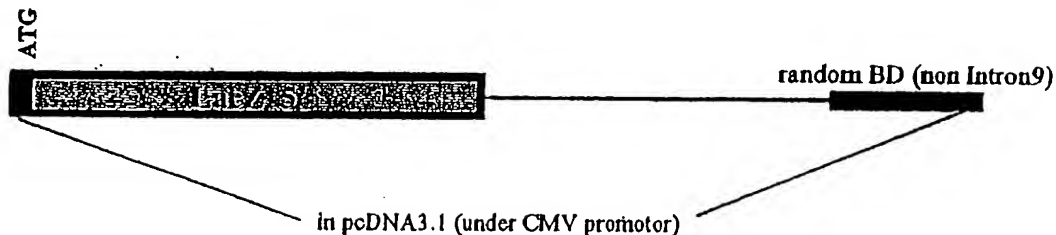


FIGURE 12

PLEC-PTM-5 for the introduction of the 1287ins3 mutation
in 293T cells

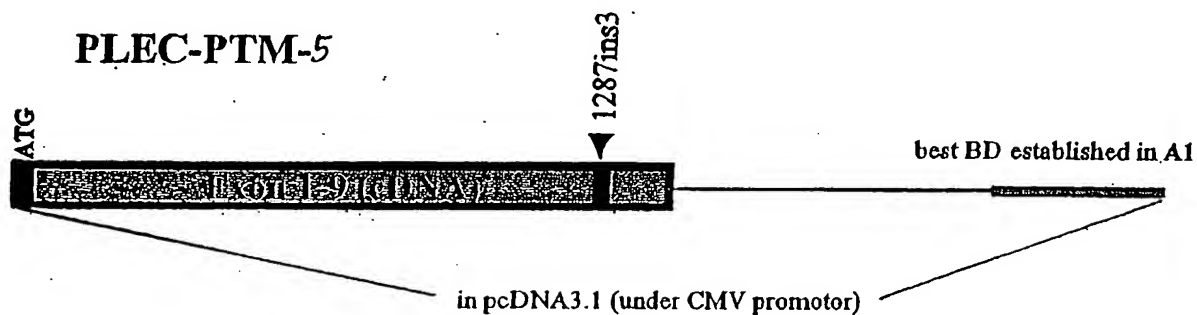


FIGURE 13

PLEC-PTM-6 for the repair of the 1287ins3 mutation
in plectin deficient patient cells

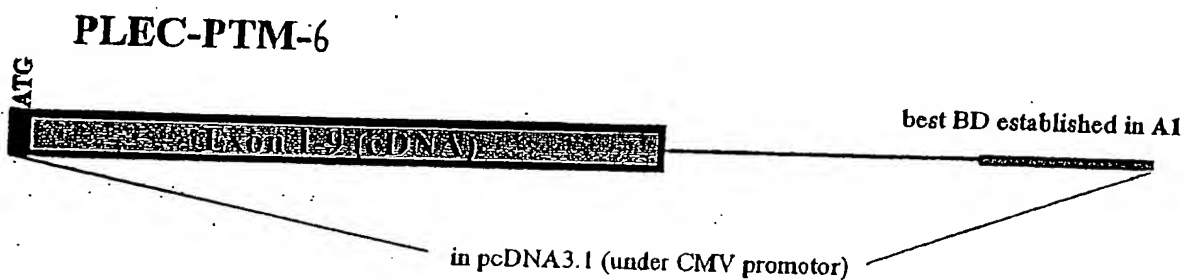


FIGURE 14A

Trans-splicing Strategy for Col17A1 Gene (GABEB/generalized atrophic benign epidermolysis bullosa)

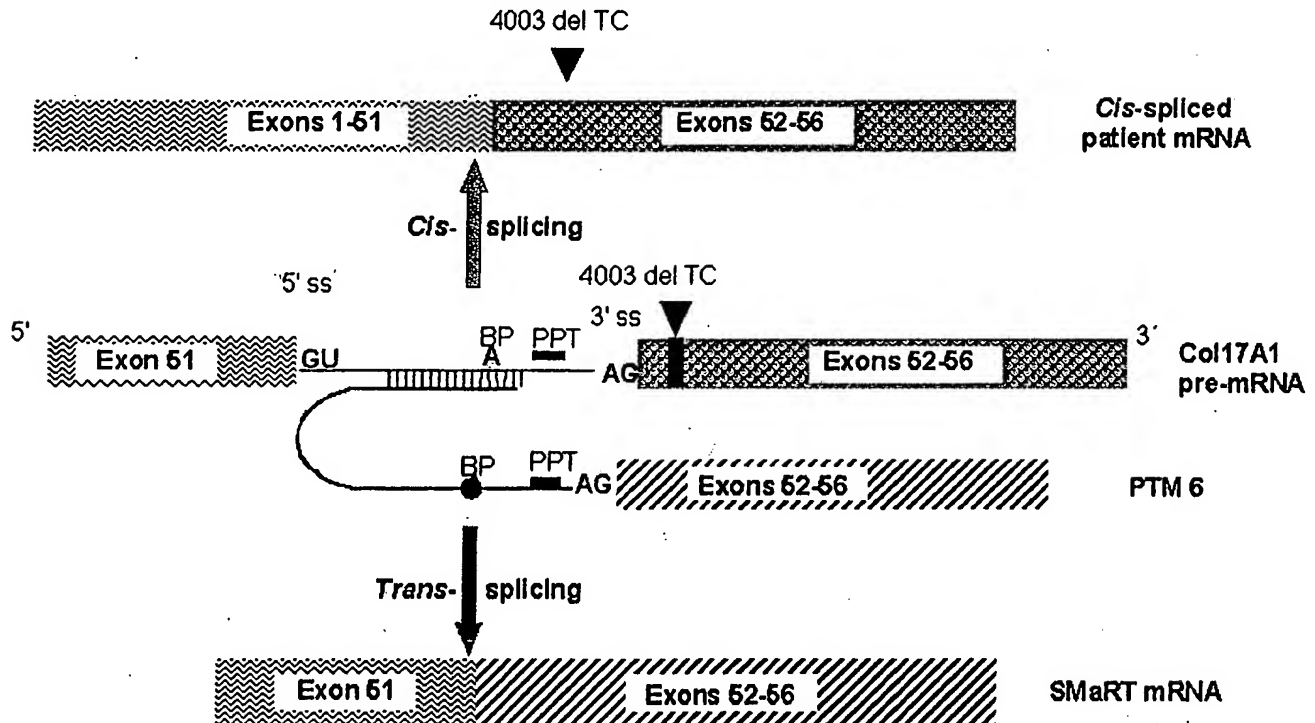
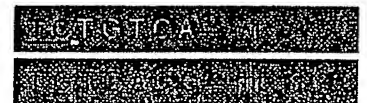
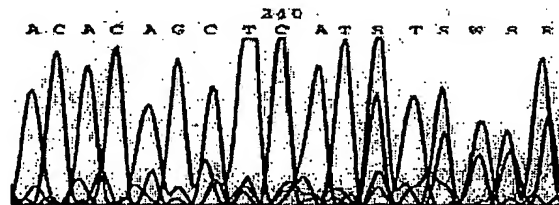
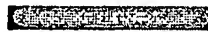


FIGURE 14B

Semi-nested RT-PCR with BPAG2- primer 51-1F, 53-1R and 52-1R

RT-PCR



F1
B1
C

FIGURE 14C

TOPO Cloning + Nla III digest

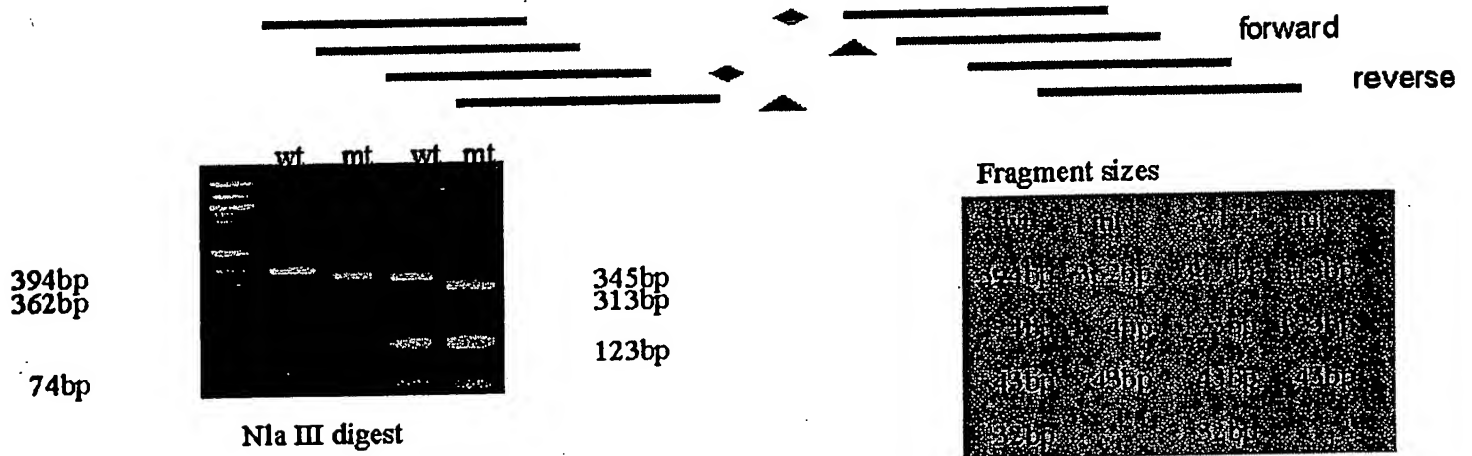


FIGURE 14D

Sequencing of mutant and
wildtype clones confirmed the
correct *trans*-splicing of PTM6
into GABEB cells

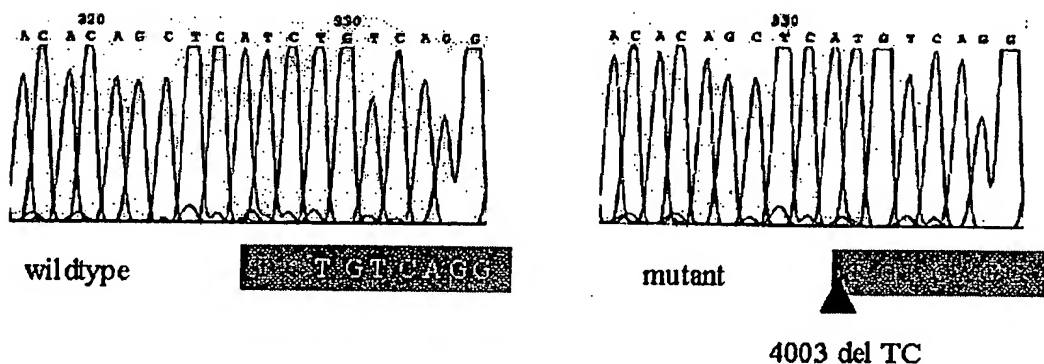


FIGURE 14E

Analysis of 100 clones

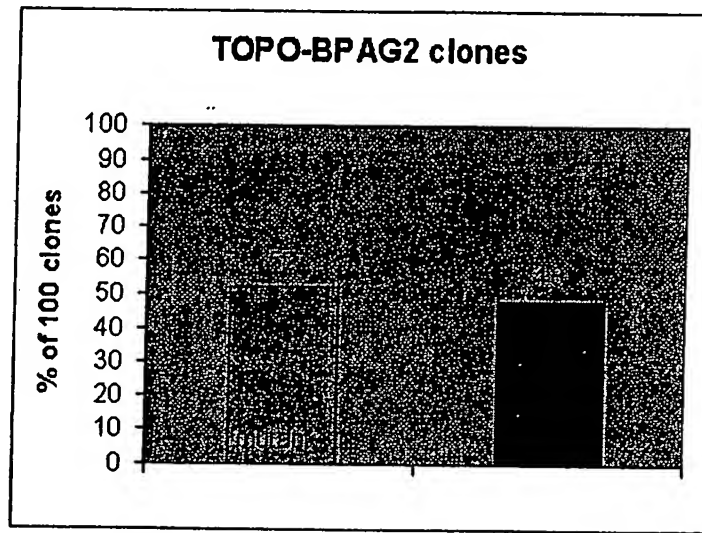


FIGURE 15

Immunofluorescence of PTM6- transfected GABEB cells

GABEB
non-transfected



GABEB
PTM6 transfected, 3 days



FIGURE 16A-B

Trans-splicing strategy for COL7A1 gene (Dystrophic epidermolysis bullosa)

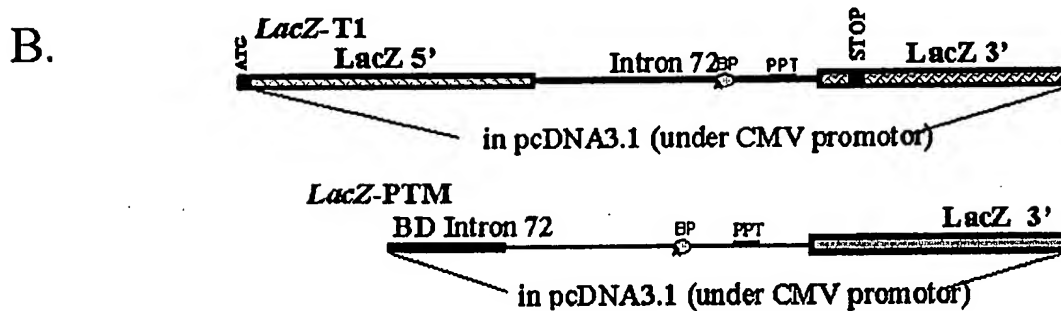
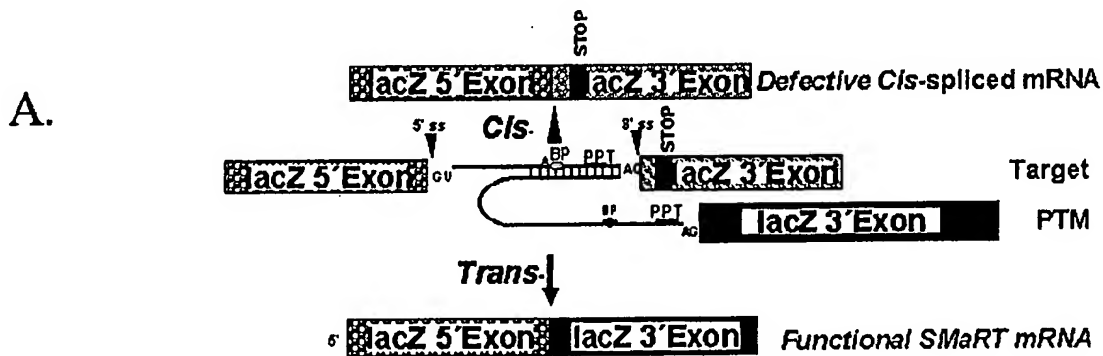


FIGURE 17

β -gal Assay of a Cotransfection of Col7 Target T1 and PTM 8, 9, 10 in 293T cells

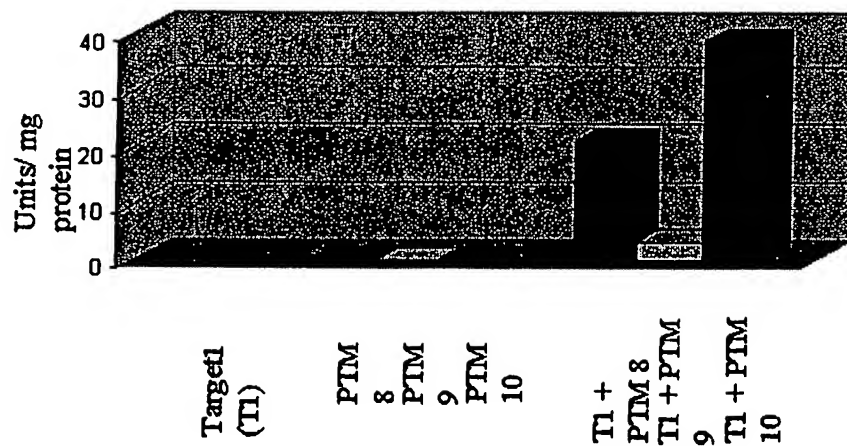
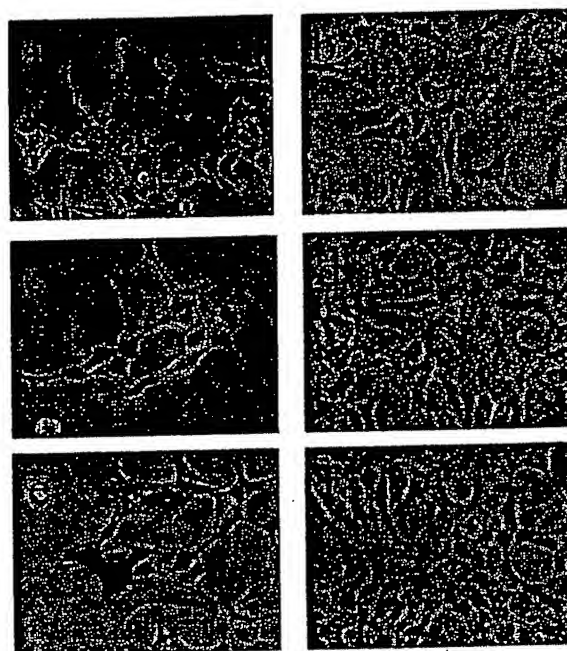


FIGURE 18

Beta-gal staining of Col7 PTM transfected human 293T cells



left panel: (a) Target + PTM8, (c) Target + PTM9, (e) Target + PTM10;
right panel: corresponding control PTM without Target.